

Message from the Director



As the Director of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), I am pleased to present this annual report highlighting the research efforts and programs supported by the Institute. The NIDDK has a broad research responsibility that includes some of the most common, debilitating, and costly conditions affecting Americans. These conditions include diabetes and other endocrine and metabolic diseases, such as cystic fibrosis; liver disease and other digestive diseases and conditions, such as inflammatory bowel disease and irritable bowel syndrome; nutritional disorders and obesity; kidney diseases, such as polycystic kidney disease; urologic diseases and conditions, such as interstitial cystitis/painful bladder syndrome and prostatitis; and hematologic diseases, such as sickle cell disease.

The 15th edition of this report illustrates recent NIDDK-supported scientific advances, such as:

- Insight into how a risk gene for type 1 diabetes might exert its effect, with potential therapeutic implications
- Preliminary evidence that bariatric surgery may be more effective than current non-surgical treatments for type 2 diabetes, after a year of treatment, in individuals with mild to moderate levels of obesity
- Finding that rare mutations reduced people's risk of type 2 diabetes by halving the amount of a certain protein in their cells, suggesting therapeutics targeting the protein may be safe and effective for diabetes prevention or treatment
- Highly encouraging results from studies testing how well artificial pancreas technologies help people with type 1 diabetes improve their blood glucose (sugar) control in "real-world" settings outside the clinic
- Fundamental insights into the identity, activities, and impact of microbes present in the gut, and how changes in this "microbiome" early in life may have important clinical implications for treating or preventing malnutrition, obesity, and other diseases
- Identification of host and microbial factors associated with inflammatory bowel disease that could pave the way to new treatment approaches
- A potential new model for predicting liver toxicity of drugs before they are tested in humans
- Discovery of molecular mechanisms that turn calorie-storing white fat into calorie-burning "beige" fat, which may open the door to new ways of treating obesity and improving metabolic health
- New data on the impact of *APOL1* gene variants on risk of kidney disease progression and cardiovascular disease in African Americans
- A new understanding of why a cystic fibrosis treatment strategy is not working as expected, providing important insights in the quest to improve therapy for people with the most common mutation causing this disease
- Demonstration that long-term use of a drug combination dramatically reduces the risk of recurrent urinary tract infection in children with vesicoureteral reflux
- Success in reversing sickle cell disease with a new, less aggressive blood stem-cell transplant regimen that could be an option for treating older or sicker adults with the disease

This report also includes personal stories of those who have given time and effort to participate in NIDDK-sponsored clinical research. A woman whose liver had been damaged by the hepatitis C virus describes the road she has traveled with family and scientists on the way to living virus-free. A man who has lived with type 1 diabetes for over 50 years joyfully shares how his life has been transformed by an islet transplant. A Marine with polycystic kidney disease describes

his determination to make a healthy future for himself and others, including participating in a clinical trial even while deployed overseas. A teenager and her mother discuss how getting a diagnosis and the right treatment for a rare metabolic disorder has changed her life for the better.

The NIDDK is continuing efforts to ensure that knowledge gained from its research advances is disseminated to health care providers, patients, and the general public. Such efforts include the Institute's education programs: the National Diabetes Education Program and the National Kidney Disease Education Program. Recent activities of both these programs are highlighted in this publication. Additionally, the Weight-control Information Network, the National Diabetes Information Clearinghouse, the National Digestive Diseases Information Clearinghouse, and the National Kidney and Urologic Diseases Information Clearinghouse develop and distribute science-based information on diseases and disorders within the NIDDK mission. Several hundred brochures, fact sheets, and publications are available to patients, health care providers, and the public both in printed format and on the NIDDK website. I invite you to visit us at www.niddk.nih.gov

The efforts featured in this publication reflect the core mission of the NIDDK, including the Director's guiding principles:

- Maintain a vigorous investigator-initiated research portfolio
- Support pivotal clinical studies and trials
- Preserve a stable pool of talented new investigators
- Foster exceptional research training and mentoring opportunities
- Ensure knowledge dissemination through outreach and communications

More information on how the NIDDK's activities support these core values can be found in the "NIDDK Funding Trends and Support of Core Values" section at the end of this report and on our website at www.niddk.nih.gov

If you have visited the NIDDK online in the past year, you may have noticed some changes. On December 16, 2013, the NIDDK launched its dynamic new website. Created through the dedicated labor and teamwork of staff across the NIDDK, this redesigned website enables us to continue to help our scientific community, patients, and the public. The NIDDK's health information has always been well received by more than 25 million online users annually. Our major goal with the revised website is to provide the same level of clear and current information for researchers. Our research community can now more easily access information about current funding and research training opportunities, NIDDK personnel, and research resources. The site also highlights important NIDDK-supported basic, clinical, and translational advances in the wide range of acute and chronic diseases and conditions in our mission, and includes a quarterly "Director's Update," which provides updates on NIDDK-supported research, health information and education programs, and other news of interest at the NIDDK and the National Institutes of Health (NIH).

This report reflects only a fraction of the immense body of NIDDK-funded research performed by basic scientists, clinical investigators, and patient volunteers. Moving forward, we remain committed to supporting these important areas of research and translating scientific discoveries into improvements in the health and quality of life of all people.



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